Subject:	Fabrazyme (agalsidase beta)		
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Overview

This document addresses the clinical indications for Fabrazyme (agalsidase beta), a biosynthetic form of human alpha-galactosidase A enzyme. Fabrazyme is an enzyme replacement therapy (ERT) approved for the treatment of individuals with a lipid storage disorder called Fabry disease.

Fabry disease is an X-linked lysosomal (lipid) storage disorder related to a deficiency of the enzyme alpha-galactosidase A (α-Gal-A, also known as ceramide trihexosidase) required to metabolize lipids. Signs and symptoms of Fabry disease include burning sensations in the arms and legs (that worsens with exercise and hot weather), small, non-cancerous, raised reddish-purple blemishes on the skin, and clouding of the corneas. Other symptoms include decreased sweating, fever, and gastrointestinal difficulties. Lipid storage may lead to breathing and digestive problems, impaired circulation, and increased risk of cardiomyopathy, cerebrovascular accidents, and renal failure.

The American College of Medical Genetics (ACMG) (2011) and National Society of Genetic Counselors (NSGC) (2013) recommend screening for deficient α-Gal-A enzyme activity in males followed by confirmatory galactosidase alpha (*GLA*) gene sequencing. As α-Gal-A activity is unreliable in females, GLA gene sequencing should be performed for a confirmatory diagnosis.

ACMG states ERT is the standard of care for symptomatic individuals as it has shown improvements in the rate of renal dysfunction, pulmonary and gastrointestinal symptoms.

Clinical Criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Fabrazyme (agalsidase beta)

Requests for Fabrazyme (agalsidase beta) may be approved if the following criteria are met:

I. Individual has a diagnosis of Fabry disease as confirmed with either of the following (ACMG, NSGC):
   A. Documentation of complete deficiency or less than 5% of mean normal alpha-galactosidase A (α-Gal A) enzyme activity in leukocytes, dried blood spots, or serum (plasma) analysis; **OR**
   B. Documented galactosidase alpha gene mutation by gene sequencing;

AND

II. The individual to be treated has one or more symptoms or physical findings attributable to Fabry disease (ACMG), such as but not limited to:
   A. Burning pain in the extremities (acroparesthesias); **OR**
   B. Cutaneous vascular lesions (angiokeratomas); **OR**
   C. Corneal verticillata (whorls); **OR**
   D. Decreased sweating (anhidrosis or hypohidrosis); **OR**
   E. Personal or family history of exercise, heat, or cold intolerance; **OR**
   F. Personal or family history of kidney failure.

Quantity Limits

Fabrazyme (agalsidase beta) Quantity Limit

Drug	Limit
Fabrazyme (agalsidase beta) 5 mg, 35 mg vial	1 mg/kg every two weeks

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J0180	Injection, agalsidase beta, 1 mg (Fabrazyme)
S9357	Home infusion therapy, enzyme replacement intravenous therapy; (e.g., Imiglucerase); administrative services, professional pharmacy services, care coordination, and all necessary supplies and equipment (drugs and nursing visits coded separately), per diem

ICD-10 Diagnosis

E75.21	Fabry (-Anderson) disease

Document History

Revised: 09/23/2019
Document History:

- 09/23/2019 – Administrative update to add drug specific quantity limit.
- 08/17/2018 – Annual Review: Minor wording and formatting changes.

References

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